COLORADO
Department of Health Care Policy & Financing

1570 Grant Street
Denver, CO 80203

Health First Colorado (Colorado's Medicaid Program) Coverage Standards for Brineura (cerliponase alfa)

December 2020

Brineura requests will be evaluated for medical necessity and reviewed on a case by case basis for all Health First Colorado Members with a diagnosis of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2) based on the following:

1. Medical records and/or genetic testing confirm:
    a. Member has mutations in *TPP1* (tripeptidyl peptidase 1) gene AND
    b. Member has a diagnosis of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as:
        i. Tripeptidyl peptidase 1 (TPP1) deficiency
        ii. Jansky-Bielschowsky disease
    c. Member has mild to moderate disease documented by a two-domain score of 3- 6 on motor and language domains of the Hamburg Scale, with a score of at least 1 in each of these two domains

2. Member is 3 years or older at time of Brineura administration

3. Treating and prescribing provider(s) attest to the following:
    a. Physician is experienced in intraventricular administration
    b. Member or member's caregiver has been counselled on the potential risks and potential benefits of all components of treatment
    c. Treatment is 10mL (300mg) Brineura followed by 2mL of intraventricular electrolytes administered once every other week by intraventricular infusion using the appropriate Brineura Administration Kit
    d. First dose occurs at least 5-7 days after intraventricular device implantation (most recent device, if replaced)
    e. Prior to each infusion:
        i. Sample of cerebrospinal fluid is obtained for cell count and culture (to identify any device-related infection)
        ii. Pretreatment with antihistamines +/- antipyretics or corticosteroids given 30-60 min prior to the start of infusion (unless clinically contraindicated)
    f. Post infusion:

i. Monitor and assess vital signs (such as, blood pressure and heart rate); signs and symptoms of anaphylaxis
ii. ECG performed at least every 6 months

g. Treating and prescribing provider(s) attest to the following:
   i. Member will be assessed by the following exam scales or other validated assessment tool at baseline and during all subsequent office visits, completed at least every 6 months AND will provide results to Health First Colorado via email (HCPF_PharmacyPAD@state.co.us).
      1. Baseline clinical and neurological exam results will be provided including the name, score and date of the assessment tool
         a. Motor and language domains of the Hamburg CLN2 Clinical Rating Scale (efficacy for the Language domain cannot be established)
   ii. Member is able and willing to be compliant to treatment and treatment requirements

4. Member must *not* have any of the following:
   - Any sign of acute, unresolved infection on or around the device insertion site, suspected or confirmed CNS infection
   - Any acute intraventricular access device related complication
   - Ventriculoperitoneal shunts
   - Any other inherited neurologic disease
   - Any contraindication to MRI scans or neurosurgery
   - Pregnancy

5. Initial approval may be approved for 7 months to allow for additional, on treatment clinical and neurological exam results at six months. Subsequent approvals may be approved for 12 months. For reauthorization (after 7 or 12 months), if there has been a decline in motor domain, noted by ≥ 2 point loss in the motor domain of the CLN2 CRS, rationale and additional supporting documentation is provided.

Above coverage standards will continue to be reviewed and evaluated for any applicable changes due to the evolving nature of factors including disease course, available treatment options and available peer-reviewed medical literature and clinical evidence. If request is for use outside of stated coverage standards, support with peer reviewed medical literature and/or subsequent clinical rationale shall be provided and will be evaluated at the time of request.

References:

1. Schulz A, Ajayi T, Specchio N, et al. Study of Intraventricular Cerliponase Alfa for CLN2 Disease. N Engl J Med. 2018 May 17;378(20):1898-1907. doi: 10.1056/NEJMoa1712649.

2. ClinicalTrials.gov. A Phase 1/2 Open-Label Dose-Escalation Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Efficacy of Intracerebroventricular BMN 190 in Patients With Late-Infantile Neuronal Ceroid Lipofuscinosis (CLN2) Disease. 2019; https://clinicaltrials.gov/ct2/show/NCT01907087. Accessed December 16, 2020.

3. Brineura [package insert]. Novato, CA; BioMarin Pharmaceutical Inc, Inc. 2020.